INSIST! ACUTE MYELOID LEUKEMIA (AML)

Program Resource Guide



Essential Testing in AML Care

Molecular Testing (genetic or biomarker testing): Laboratory testing that identifies certain gene mutations, proteins, chromosomal abnormalities and/or other molecular changes that are unique to an individual's disease. In cancer, it may be used to evaluate treatment or to make a prognosis.



Targeted Therapy: A type of personalized medicine that works by blocking specific mutations and by preventing cancer cells from growing and dividing, without affecting normal cells.

Genetic Testing Methods

Karyotyping: Testing of blood, bone marrow, or tissue in order to identify changes in chromosomes.

Fluorescence In Situ Hybridization (FISH): A chromosome test used to identify specific genes or chromosome changes.

Next-Generation Sequencing (NGS): Technology to sequence DNA or RNA to identify genetic variations associated with diseases or other biological phenomena.

Common AML Mutations

IDH (Isocitrate Dehydrogenase) Mutations:

Mutations in IDH1 or IDH2 are detected in approximately 20 percent of patients with AML.

FLT3 Mutation: FLT3 stands for Fms-like tyrosine kinase. This gene mutation occurs in approximately 30 percent of AML patients.

NPM1 (Nucleophosmin-1) Mutation: The most common molecular mutation identified in adult AML.

TP53 (tumor protein 53) Mutation: A gene that helps stop the growth of tumors. TP53 is the most frequently mutated gene in human tumors. TP53 mutation rates are low in AML.

Approved FLT3 Inhibitors

FLT3 Inhibitor Therapy

- Gilteritinib (Xospata)
- Midostaurin (Rydapt)
- Sorafenib (Nexavar)

Approved IDH Inhibitors

IDH1 Inhibitor Therapy

- Ivosidenib (Tibsovo)
- Olutasidenib (Rezlidhia)

IDH2 Inhibitor Therapy

Enasidenib (Idhifa)

Venetoclax (Venclexta): Inhibitor therapy that targets the Bcl-2 protein. Venetoclax is being used in combination with hypomethylating agents (azacitidine [Vidaza] or decitabine [Dacogen]) to treat older AML patients who are not candidates for intensive induction therapy.

Glossary Terms

AGILE Study: Global, Phase III, multicenter, double-blind, randomized, placebo-controlled clinical trial to evaluate the efficacy and safety of AG-120 (ivosidenib) + azacitidine vs placebo + azacitidine in participants with previously untreated acute myeloid leukemia (AML) with an IDH1 mutation.

Bone Marrow Biopsy: Procedure that involves collecting a small sample of bone marrow, usually from the hip bone, in order to be examined by a laboratory. This procedure is used to confirm a diagnosis and may be used to monitor the disease over time.

Menin Inhibitors: A novel class of targeted therapies showing promise in the treatment of acute leukemias with them NPM1 mutation or the KMT2A mutation.

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